



Background to the Panel

- Managed entry agreements are growing in popularity worldwide, especially for pharmaceuticals
- Application of these schemes to medical devices may raise some additional challenges
- Such schemes are often highly dependent on the local context and experience may not be simply transferable to other settings

Panelists

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Overseas Experience of Managed Entry Agreements

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Outline of Presentation

- What are managed entry agreements?
- What experience do we have?
- Are there issues specific to medical devices?
- What lessons can Japan learn

Need for Managed Entry Agreements

- Decision-makers may feel that a new technology has promise, but have uncertainty about:
 - long term outcomes, and/or
 - the size of the overall financial commitment
- A managed entry agreement (MEA) allows the technology to be reimbursed, while limiting the financial risk to the decision-maker
- MEAs can represent a 'win' for patients, the decision-maker and the manufacturer

Types of Agreement*

- *Performance-based schemes*
 - useful when the main need is to reduce uncertainty about the benefits of the technology
- *Finance-based schemes*
 - useful when the main need is to address issues of affordability concerning the technology

* *In many situations these two components are inter-related. For example, a reduction in the price of one of the technologies being compared can change the level of uncertainty concerning which technology is cost-effective*

When Should we Consider Outcomes-Based Agreements?

- Outcomes-based schemes are most useful when there is uncertainty in clinical or economic outcomes
- Sources of uncertainty include:
 - long term clinical outcomes (eg maintenance of clinical effect or to validate a surrogate endpoint)
 - performance of the technology in different patient sub-groups
 - clinical or organizational response to the new technology

Note: If the main issue concerns the cost or affordability of a technology, outcome-based schemes are a wasteful way of addressing this issue

Common Terminology for Outcomes-Based Agreements

- ‘Coverage with Evidence Development’ (US)
- ‘Only with Research’ (UK)
- ‘Field Evaluations’ (Canada)
- ‘Risk-Sharing Schemes’ (Many countries)
- ‘Patient Access Schemes’ (UK)
- ‘Performance-Based Risk-Sharing Arrangements’ (ISPOR Task Force)
- ‘Pay for performance’ (Australia)

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ISPOR TASK FORCE REPORTS

Performance-Based Risk-Sharing Arrangements—Good Practices for Design, Implementation, and Evaluation: Report of the ISPOR Good Practices for Performance-Based Risk-Sharing Arrangements Task Force

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Perceived Benefits of PBRsAs

- Potential to enhance coverage decisions and strengthen existing evidence bases on the benefits and costs of new technologies.
- Enable payers to participate in the research process.
- Allow hospitals and clinicians to monitor more closely procedures being performed and manage costs until benefit is substantiated.
- Encourage industry to generate the data needed to support the value claims of their innovations.
- Allow earlier access for patients to potentially valuable treatments than they might otherwise be granted.

National Approaches to CED: Examples from Canada, US, UK

Country	Name of Program	Aims	Year Established	Technologies Included	Actors Involved	Funding Sources	Examples of CED
Canada	Conditionally-funded Field Evaluations	Assess real world performance; address outstanding uncertainty about benefits/costs; improve coverage decision making.	2003	Non-drug technologies (devices and procedures)	OHTAC, PATH, THETA, ICES, Ontario Health Ministry	OHTAC funds the evaluations; Ministry funds device (or procedure) if not yet insured.	Over 40 studies to date. Examples include: • PET • DES • CT angiography • Sleep apnea device
US	CED	Allow greater flexibility in coverage determinations; link coverage to efforts to generate evidence needed to gain greater certainty on the benefits and harms of particular technologies.	2006	Procedures, devices, and drugs.	CMS	No standard or requirements for funding. Public agencies, such as NIH or AHRQ may fund studies, as well as manufacturers, medical associations, or academic research groups.	Over 15 studies to date. Examples include: • PET • ICDs • Lung Volume Reduction Surgery • Angioplasty and stenting • Transcutaneous Electrical Nerve Stimulation
UK	Only in Research (with limited use of Approval with Research)	Provide coverage to interventions not yet supported by sufficiently robust evidence, while additional data is collected.	1999	Procedures, devices, and drugs.	NICE, NIHR	No standard or requirements for funding. NIHR or manufacturer may fund study.	Over 25 studies to date. Examples include: • PET • ICDs • Metal-on-metal hip implants • Drainage, irrigation and fibrinolytic therapy (DIFT) • Laparoscopic surgery

Drug Eluting Stents in Ontario

- The generalisability of existing randomised controlled trials (conducted in the US) was questioned
- A pragmatic registry of all patients receiving DES was established, in order to conduct a 'field evaluation' (between 2003-2004)
- Coverage was provided for the stents provided under the registry
- Found that DES was more effective only in patients at high risk of stenosis (those with diabetes, or particularly long or narrow lesions)
- This represented about 30% of the whole patient population
- Argued that this policy saved between \$35-58 million, as compared with the potential uncontrolled adoption of DES

Key Challenges in Performance-Based Agreements

- Establishing a clear framework for applying PBRsAs (e.g. deciding when they are appropriate).
- Identifying and applying appropriate research methods (e.g. RCTs, observational studies).
- Involving all the relevant parties (e.g. manufacturers, health providers, professional groups).
- Funding and conducting the research.
- Determining appropriate coverage arrangements based on the research findings.

Can Observational Studies Help Us Estimate Relative Treatment Effect?

- Writing in the context of the revisions to the Cancer Drugs Fund in the UK, Grieve et al argue that simple randomized clinical trials, using routinely collected data are required

Grieve R et al British Medical Journal 2016;354:i5090

- However, the ISPOR Task Force on Prospective Observational Studies argue that ‘well-designed and well-executed observational studies can provide evidence of causal relationships’

Berger M et al Value in Health 2012;15: 217-230

Complexity and Cost of Arrangements

- Complexity and cost is a common reason for outcomes-based agreements not being pursued
- In most jurisdictions the manufacturer is expected to bear the cost of data collection and monitoring (although this is up for discussion)
- More complex schemes may result in less transparency about the price being paid for the drug or other health technology

Connecting Decisions to the Outcomes Obtained

- A common concern of manufacturers is that there is often uncertainty regarding the policy decisions following outcomes-based schemes
- Agreements are more likely to succeed if the consequences for pricing and reimbursement are set out clearly in advance, preferably in a written agreement

Key Success Factors for PBRsAs

- When there is uncertainty about clinical or economic outcomes
- When outcome targets can be clearly defined and measured
- When performance-based arrangements are not excessively complicated or costly
- When the timelines are reasonable
- When reimbursement and/or pricing decisions clearly follow the outcomes obtained

Drummond MF (2015) *Eur. J Health Econ* DOI 10.1007/s10198-015-0683-z

Are MEAs a Way Forward for the Pricing and Reimbursement of Health Technologies?

- The answer depends on the answers to the following questions:
 - Can the current pricing system be reformed?
 - Are decision-makers flexible?
 - Is there a capacity/willingness to conduct studies?
 - What are the local views on price transparency/ secrecy?

Conclusions on Outcomes-Based Agreements

- They are clearly worth considering when the conditions are right
- However, the devil is in the detail, so payers and manufacturers need to consider carefully whether an outcomes-based agreement is the best way forward